Specialised Healthcare Alliance
FOR EVERYONE WITH RARE AND COMPLEX CONDITIONS

Valuing rarity: Assessing the impact of the NICE methods review

Executive summary

The National Institute for Health and Care Excellence (NICE) concluded a review of its methods and processes in January 2022, marking the end of a long period of consultation. This resulted in notable changes that affected the assessment of rare disease treatments, including the introduction of the 'severity modifier' to give additional weight to health benefits in the most severe conditions and the acceptance of higher degrees of uncertainty for treatments where it is difficult to generate evidence.

Since then, some progress has been made in enabling access to innovative new medicines for rare diseases, including the use of an innovative payment model to facilitate the first approval of a rare disease treatment through the Innovative Medicines Fund in June 2024.

However, **many rare disease patients are still waiting too long to access new treatments**, and in some cases are missing out all together on potentially life-changing treatments available in comparative European countries.

The Specialised Healthcare Alliance (SHCA) has developed this report to reflect on progress since 2022 in enabling access to treatments for patients with rare diseases, informed by engagement with our members and corporate supporters regarding their experiences of working with NICE as part of the assessment of rare disease treatments.

A survey of our membership and corporate supporters found that:

11 of 12 respondents do not consider NICE's methods and processes to be suitable for the appraisal of rare disease treatments

10 of 12 respondents think that NICE's criteria for determining whether a treatment should be assessed through the Highly Specialised Technologies (HST) programme are inappropriate for the assessment of rare disease medicines

8 of 12 respondents believe that NICE's application of the routing criteria are not sufficiently transparent

Based on these findings, **the SHCA is calling for a specific focus on rarity in future modular updates, alongside a *NICE Listens* exercise to understand the social value associated with treating rare diseases**. This report recommends that:

⇒ NICE uses its patient involvement strategy to review patient groups' experiences working with NICE as part of rare disease technology appraisals, including reviewing the process of submitting evidence

- ⇒ The upcoming review of the HST programme routing criteria includes consultation with the rare disease community on its appropriateness for the assessment of very rare diseases
- ⇒ An analysis of the effectiveness of the new severity modifier is carried out, to determine its appropriateness for the assessment of rare disease treatments

The SHCA is a coalition of over 140 charities and corporate supporters which advocates on behalf of people living with rare and complex conditions.

Introduction

The National Institute for Health and Care Excellence (NICE) is the independent assessment body with responsibility for taking decisions on which new treatments and technologies should be made available by the NHS in England.

In January 2022, NICE published its updated programme manual setting out changes to its methods and processes.[1] The objective of the review was to ensure NICE keeps pace with developments in science, enabling it to evaluate new technologies fairly, efficiently and robustly.

While the review resulted in the introduction of some changes of note, such as the creation of the severity modifier and the acceptance of higher degrees of uncertainty for treatments where it is difficult to generate evidence, NICE rejected introducing further changes to the assessment of rare disease treatments, informed by an assessment that there was no societal preference for additional value to be attached to rare diseases.

Following the review, the BioIndustry Association (BIA) published a report exploring societal views on rare diseases, which found that the public does believe that a distinctive and alternative approach should be adopted for making funding decisions about treatments for rare diseases. It called on NICE to utilise its *NICE Listens* programme to undertake its own primary research to assess whether it needs to update its previous conclusions.[2] Building on the findings of that report, the Specialised Healthcare Alliance (SHCA) has examined the impact of the changes introduced as part of the review on access to rare disease treatments.

To examine the impact of the changes that NICE introduced and to assess the impact they have had on rare disease technology appraisals, we disseminated an open survey to the SHCA's membership and corporate supporters to collect quantitative and qualitative feedback on their experiences working with NICE. 12 responses were received: 6 from corporate supporters and 6 from charity members. 10 of the 12 respondents had participated in at least one NICE technology appraisal under the new methods and processes. Alongside that, we also held some one-on-one conversations to probe the findings of the survey further.

Based on those findings, this report focuses on:

Unpacking SHCA members and supporters' experiences working with NICE on rare disease appraisals

Bridging the gap between the Single Technology Appraisal (STA) pathway and Highly Specialised Technology (HST) pathway

Evaluating the impact of the severity modifier in enabling access to rare disease treatments

Exploring the case for prioritising rarity in NICE's modular updates

Recommendations for change

The SHCA has welcomed NICE's constructive approach to engagement and the ongoing work being carried out by NICE analysing the impact of its methods and processes review, including the application of the severity modifier and the HST routing criteria. We look forward to continuing to work with NICE to consider how the recommendations made in this report can be implemented and integrated into that ongoing work.

SHCA members and supporters' experiences working with NICE on rare disease appraisals

Background

The UK Rare Diseases Framework defines a rare disease as occurring in fewer than 1 in 2,000 people, affecting over 3.5 million people in the UK.[3] A very rare disease is defined by NICE as occurring in fewer than 1 in 50,000 people, affecting fewer than 1,100 people.[4] Treatments for very rare conditions that meet a set of strict criteria are assessed through the HST pathway, whereas all other rare disease treatments are assessed through the STA pathway.[4]

For a rare disease treatment to be approved through the STA programme, NICE needs to find that it is cost effective in consideration of how a disease affects people's quality of life and the length of life they will gain as a result of an intervention, expressed as quality-adjusted life years (QALYs). Treatments that cost between £20,000 and £30,000 per QALY may be deemed as cost effective under an STA if they meet certain criteria. The HST pathway has a much higher cost-effectiveness threshold of £100,000-£300,000 per QALY, in recognition of the challenges that exist in developing treatments for ultra rare conditions and to encourage innovation in this area when there are challenges in generating an evidence base that is robust enough to bring a product to market.

The Rare Diseases Framework acknowledges the challenges NICE and other HTA bodies face in evaluating rare disease treatments, due to small patient populations and accompanying uncertainty. Additionally, as approved medicines are only available for around 5% of rare diseases, many rare diseases have no current treatment alternatives. The absence of suitable comparators makes it harder to demonstrate both the impact of a treatment on the quality and length of life and the cost-effectiveness of a new treatment compared to the very low-cost status quo.[5]

To assess the impact of the changes introduced by NICE, we asked SHCA members and corporate supporters to share feedback on their experiences working with NICE in the assessment of a rare disease treatment and specific challenges encountered. This section summarises common themes identified.

Communication with NICE

11 of 12 of SHCA members and supporters surveyed do not consider NICE's methods and processes to be suitable for the appraisal of rare disease treatments.

Processes for engagement with charities, clinicians and companies

In 2023, the SHCA and Genetic Alliance UK published a report that captured SHCA members' experiences of patient involvement in NICE decision making.[6] Challenges in communication with NICE during technology appraisals were echoed in this report. Some members felt that, once scoping had been completed, developments at committee stage were not always well communicated and charities often did not have the resource to engage and challenge effectively.

One corporate supporter told us that for the second committee meeting of one appraisal, the NICE External Assessment Group (EAG) report and meeting slides were only shared 48 hours before the meeting, giving them insufficient time to effectively prepare for the meeting and structure evidence. The short window patient groups have to respond to consultations on EAG reports also creates challenges, given the limited resources of rare disease charities and the highly technical nature of these reports.

Another member explained that whilst patient advocates are told they do not need to have expertise on how NICE appraisals work to give evidence, committees often question them as if they are experts, and the shortened format of committee meetings prevents them from effectively sharing their experience and comments. We heard how in one case this shortened format meant a clinician was cut off whilst giving evidence.

"At committee stage the patient advocate had very little time to respond to or interact with the committee. This really impacted their and clinicians' ability to respond to uncertainty."

SHCA members welcome the recently published outline of NICE's patient involvement strategy, including the commitment to introduce a new payment that fairly and transparently compensates people for their involvement and engagement. It is important that final strategy considers how the format and structure of committee meetings can be adapted to empower patient advocates to share their insights and experiences effectively. Patient groups should also have an opportunity to share feedback on working with NICE on a technology appraisal after final guidance is published, to provide reflections on what worked well and where final support was required.

Managing uncertainty

The EQ5D is the tool NICE uses to assess quality of life and inform calculations about the QALY gain associated with a treatment.

Managing uncertainty is one of the key challenges faced by HTA bodies in assessing rare disease treatments, due to the small patient populations, limited available data and lack of comparator treatments. In recognition of this, the final NICE programme manual states that: "*In circumstances when evidence generation is difficult (for example, for rare diseases), when there is insufficient data to assess whether the EQ-5D adequately reflects changes in quality of life, evidence other than psychometric measures may be presented and considered to establish whether the EQ-5D is appropriate*".[1]

However, responses to our survey were unanimous in documenting challenges working with NICE to resolve uncertainty, with limited flexibility applied to rare disease treatments and a lack of acceptance of the challenges that exist around evidence generation. Members told us they found committees often push for the lower end of estimates on the cost per QALY thresholds, with rare disease treatments disadvantaged by the absence of suitable comparators making it difficult to prove clinical and cost effectiveness. The lack of a specific framework for when flexibility should be applied by committees means that there is inconsistency in application and many rare disease treatments are impacted negatively as a result.

"The evidence expectations and thresholds of NICE are not commensurate with the complex realities of conducting clinical trials in these small populations."

Understanding the burden of disease

Many of our members highlighted how NICE's methods, both for STA and HST, fail to recognise the wider burden of rare diseases on patients and society – including the seriousness of the condition, carer and wider family burden, and societal and financial impacts. QALY thresholds are not determined in recognition of the impact a treatment may have in preventing irreversible disability and long term complex medical needs. It was also noted that new symptoms of very rare diseases often emerge after clinical trial design, but this is not captured in appraisals where trials are judged against original end points – so the role of patient advocates is particularly important in bringing this impact to light.

We heard that whilst the HST framework acknowledges the impact of a treatment on carers and society, in reality their role in the evaluation process is limited. The ABPI has highlighted how carer quality of life data is rarely submitted because evidence is often not available, and where it is, it is not always accepted by NICE.[7] Companies should be encouraged to generate and submit this evidence where a treatment may have an impact on carer quality of life.

One member also noted that for very rare conditions, in assessing a treatment NICE assumes bereaved families will recover from grief after a short period of time, which does not reflect the reality of grief as an ongoing experience and there should be consideration of how NICE can accept this kind of evidence.

Commercial negotiations with NHS England

Due to the challenges created by the STA pathway, many rare conditions receive a negative interim Appraisal Consultation Document (ACD), before commercial negotiations then begin with NHS England (NHSE). We heard how these negotiations create an unsatisfactory situation for patients, where NHSE and the manufacturer go into a negotiating 'tunnel' that can take months to resolve and patient groups no longer have input into the process. The patients they represent then suffer from the distressing emotional impact that an interim negative recommendation creates, as well as the long periods that pass without an update on how commercial negotiations are progressing. One member called for NHSE to be more transparent on commercial processes and provide a way for patient groups to respond if negotiations are stalling or failing.

"Once you get to the NHSE negotiation after the initial NICE recommendation everything goes dark and there is no way of understanding what is going on."

One resolution to some of the above challenges could come through increased utilisation of the Innovative Medicines Fund (IMF), through which the first treatment was approved in June 2024, two years on from its introduction. It is important that NHSE reviews the factors behind the underutilisation of the IMF, including the entry and exit criteria, and sets out when and how the enhanced commercial flexibilities introduced in the 2024 Voluntary Scheme for Branded Medicines Pricing, Access and Growth (VPAG) can and should be offered.

Bridging the gap between the Standard Technology Appraisal Pathway and Highly Specialised Technology Pathway

NICE's HST programme was introduced in 2013 to create a new pathway for evaluating ultra-orphan medicines. The HST programme uses a cost-effectiveness threshold of £100,000-£300,000 per QALY, compared to the £20,000-£30,000 per QALY threshold used in the STA process.[8] Entry into the HST pathway is limited by a strict set of criteria, and in the year leading up to June 2024, only two treatments were assessed via this pathway. The entry criteria for the HST programme are that:

1. The condition the drug is treating must be considered very rare (with a prevalence of less than one in 50,000)
2. No more than 300 people in England are eligible for the drug in the licensed indication and no more than 500 across all indications
3. The very rare disease significantly shortens or significantly impairs the quality of life
4. There are no other satisfactory treatment options, or the technology is likely to offer sufficient benefit over existing treatment options

As a result of the criteria, many rare disease medicines – and in some cases even medicines for ultra rare diseases – fall into the STA process, which is designed for more common conditions. We asked

members and supports to explain their experiences working with NICE at the topic selection stage of a technology appraisal and their views on the transparency of NICE's application of the criteria.

NICE topic selection

10 of 12 SHCA members and supporters surveyed said the HST entry criteria were not appropriate whilst 8 said the application of the criteria was not sufficiently transparent.

SHCA members and supporters recognise the need for strong qualifying criteria for the HST programme, given the extent of the additional flexibility on cost-effectiveness that it provides. However, a common theme from our engagement was the subjective application of the criteria, with many examples provided of disagreements between clinical experts and the NICE topic selection oversight panel.

One of the factors that has led to these disagreements is the use of subjective terms within the criteria such as 'satisfactory', 'significantly' and 'severely' that are open to interpretation. The assessment of eligible patient populations (which can be hard to determine) and the prospective impact of a treatment on quality of life are two notable examples where divergence in opinions can arise due to subjectivity.

We heard that the topic selection oversight panel's engagement with patient groups and manufacturers should be strengthened, so that evidence gaps can be highlighted and evidence of relevance to the application of the four criteria shared. It was noted in some cases, HST checklists are not published for all technologies that are submitted to HST but rerouted to STA, and in other cases the oversight panel has rejected clinical consensus on the impact of a particular condition. Whilst flexibility has been applied to the entry criteria in the past to enable routing to the HST pathway, it was felt there was a lack of transparency around when this flexibility should be applied.

As a result of these challenges, the SHCA recommends that the appeal process around STA routing is strengthened. Specific criteria should be introduced around the circumstances where appeals can be made, including, but not limited to, sharing advice on the misinterpretation of evidence and the availability of new evidence that could impact a decision.

Evaluating the HST routing criteria

Through our engagement, we heard feedback around challenges created by the application of each of the four criteria. Criteria 1 and 2 are particularly challenging for medicines with multiple indications, where the cumulative population exceeds the very low limit of 500 patients across all of a technology's licensed indications. As a result, a second indication for a very rare disease can end up being evaluated via an STA, despite the equivalent investment in research required by a manufacturer across both indications.

This can disincentivise manufacturers from launching follow-on indications and limit available treatment options for UK patients, in conditions where new treatments are desperately needed. Recent research also highlights challenges in meeting criteria 3 and 4, with 70% of therapies considered for HST found to have failed to meet criteria 4 as a result of disputes over uncertainty – specifically the extent to which a treatment being assessed offers an increase in quality of life against the current standard of care.[9]

The 2024 England Rare Diseases Action Plan stated that NICE plans to review the criteria for determining whether a medicine should be routed to its HST programme for the evaluation of very rare diseases.[10] The SHCA welcomes this review, and it is important that it includes consultation with the rare disease community on:

- Reviewing the appropriateness of the four criteria and considering whether changes are required

- Strengthening the evidence provided by the NICE topic selection oversight panel on decisions made to a route a treatment to STA where a HST application has been made
- Setting out publicly the conditions that must be met for the topic selection oversight panel to apply flexibility in its decision-making

Evaluating the impact of the severity modifier in enabling access to rare disease treatments

The NICE methods review led to the removal of the *end-of-life modifier* and the introduction of a *disease severity modifier* under the STA programme. This change was informed by evidence that the public place greater value on treatments for severe diseases than treatments at the end of life.

NICE defines the severity of a disease or condition as the future health (defined in QALYs) lost by people living with the disease or condition and receiving standard care in the NHS. Both absolute QALY shortfall (future QALYs lost from an illness receiving the current standard of care versus someone without the condition) and proportionate QALY shortfall (the proportion of future QALYs lost because of the condition) are considered in whether the modifier should be applied. Different weightings are used depending on the severity of the condition. The highest severity weighting is allocated at £51,000 per QALY, whereas treatments of medium severity are valued at £36,000 per QALY.[1]

Despite initial optimism, rare disease treatments have not benefitted from the introduction of the modifier in the way that was hoped, with many of the challenges already highlighted around uncertainty affecting the weighting under the modifier committees are likely to apply to a rare disease treatment. As a result, few medicines have qualified for a modifier. The recent multiple technology appraisal of treatments for cystic fibrosis is one of the first times the severity modifier has been used for a non-cancer medicine, helping to enable their approval.[11]

NICE has recently acknowledged that the application of the modifier is not working as intended and is consequently reviewing the impact of its methods and processes. Our report is intended to support this review, so we asked SHCA members and supporters whether they felt the severity modifier has helped to support the assessment of treatments for rare diseases.

No SHCA member or supporter surveyed indicated that the severity modifier has made it easier for NICE to issue positive guidance for rare disease treatments.

Members and supporters felt that, in principle, moving from a restrictive end-of-life modifier to one based on disease severity represents a more equitable approach that should benefit a broader range of conditions and patients. However, we heard that the 'budget neutral' terms that accompanied the introduction of the modifier has contributed to strict application of the highest severity weighting, and this has meant rare disease patients have not benefitted.

In the majority of cases where the modifier has been used, the lower weighting has been applied. For rare disease treatments that have not been routed into HST, this is not sufficient to accommodate the gap in QALY weighting that comes from being routed to STA and has a limited impact in determining whether a treatment receives a positive recommendation.

Adapting the severity modifier for rare disease treatments

We also heard how rare disease treatments can be less likely to achieve the higher weighting than other conditions; young patients living with a severe and debilitating disease that is not life threatening but is lifelong may have a lower proportionate QALY shortfall, whereas older patients with the same disease may have a lower absolute QALY shortfall. It was also noted that, whilst NICE indicated only a small number of treatments that previously benefited from the end-of-life modifier would not benefit from

the severity modifier, the application of the lower severity modifier weighting could mean patients missing out on future innovations that would previously have been approved (the end-of-life modifier had a set weighting at £50,000 per QALY).

Discounting refers to the process where NICE 'discounts' health benefits and costs that occur in the future as they are perceived as less valuable than the benefits and costs experienced today.

The lowering of the discount rate used by NICE could help to address some of these challenges. During the review consultation exercises, NICE recommended that the discount rate be lowered from 3.5% to 1.5%, in line with the rate used across government. This was not adopted in the final manual due to budgetary constraints. As a result, it is particularly important NICE guidance to committees on applying flexibilities, including allowing relevant topics to use a 1.5 per cent discount rate to allow more patients to benefit from innovative medicines, are acted on. However, analysis from the ABPI found that whilst four topics applied for a 1.5 per cent discount rate to be applied, none were successful.[7]

To accompany the introduction of the severity modifier, NICE said it planned to carry out research to determine the societal value the public places on health benefits for severe diseases.[12] It is important that this research is now prioritised and is expanded to include an analysis of the societal value the public places on treatments for rare conditions. This will help to evaluate whether the modifier should be adapted to accommodate for the unique challenges that exist in appraising rare disease treatments.

The case for prioritising rarity in NICE's modular updates

Understanding public attitudes to the social value of rare diseases

Our engagement with members demonstrates the challenges faced by the rare disease community in securing a positive recommendation for a rare disease treatment. The strict entry criteria to the HST pathway means the vast majority of treatments are routed into STA. Although the NICE methods review recommended that increased flexibility is applied to treatments where there are high levels of uncertainty, this has not been delivered in practice. Following publication of the final programme manual, only two topics reported committees accepting a higher degree of uncertainty for rarity, despite 16 topics being orphan or ultra-orphan indications in this period.[7]

These challenges are reflected in recent statistics: in the year 2023/2024, five appraisals for rare disease treatments have been terminated due to a non-submission from the manufacturer, an increase from two rare disease terminations in the year prior the NICE final programme manual coming into effect. Whilst there are a number of reasons that contribute to a non-submission, one of them may be meeting the STA cost per QALY thresholds.[13] Meanwhile, EFPIA's 2024 data on the number of non-oncology orphan medicines available to patients in European countries shows that the UK ranked eighth in access.[14]

One solution to these challenges would be to introduce a rarity modifier, where an increased QALY weighting is applied to rare disease treatments that do not meet the HST entry criteria. The Scottish Medicines Consortium (SMC) has adapted a similar approach, accepting a greater level of uncertainty in the economic case when assessing rare disease treatments, including consideration of whether they substantially increase life expectancy and/or quality of life. However, as part of its review, NICE concluded that there was "*no evidence that society values more highly health benefits in rare diseases and that the information presented during the consultation did not provide sufficient evidence to support adding a modifier for rare diseases*."[10]

The UK Bioindustry Association's report on the social values of treating rare diseases

The BIA commissioned primary research to support NICE's requirement for more robust evidence on the social value associated with treating rare diseases.[2] This included both focus groups of deliberative public engagement and an online survey to collect quantitative data. The results found that:

- 93% of focus group participants and 80% of survey participants agreed with the statement *"people with rare diseases should have equitable access to treatments, even if this means additional costs for the NHS"*
- 82% of participants felt that NICE should evaluate the cost effectiveness of treatments for rare diseases differently than for more common diseases, considering the additional challenges of developing medicines for rare diseases
- 75% of participants believed that funding decisions for medicines to treat patients with rare diseases should be made using cost-effectiveness thresholds that fall between those for treatments for very rare diseases and those for treatments for more common diseases

Developing an alternative assessment of rare disease treatments

Based on the findings of our survey of SHCA members and supporters, this report calls for NICE to utilise the *NICE Listens* programme to undertake primary research on the social value associated with treating rare diseases. Based on the findings of that programme, future modular updates to NICE's methods and processes should consider different options for the assessment of rare disease treatments and an analysis of the accompanying implications. That should include a consideration of the case for introducing an alternative modifier for rare disease treatments.

Recommendations for change

In our survey of SHCA members and supporters, we asked for recommendations on reforms that could help to support the assessment of rare disease treatments. Building on those findings and the public consultation carried out by the BIA, we have developed a series of recommendations and suggestions for future research for NICE to consider:

SHCA members and supporters' experiences working with NICE on rare disease appraisals

⇒ NICE should carry out a review of the impact of its shortened committee meeting format and use the opportunity of its patient involvement strategy to introduce clear guidelines on patient evidence for committees, to ensure an appropriate balance between managing the length of meetings and not removing opportunities for patient input, to ensure are lessons are learned from past appraisals

⇒ In line with the recommendation made in the NICE methods and processes review, NICE should clearly set out the circumstances where alternative evidence should be considered by committees in treatments where evidence generation is challenging. As part of this, NICE should review how wider burden of disease data can be captured in a single technology appraisal

⇒ NHS England and NICE should review their engagement with patient groups during commercial negotiations, particularly where they are protracted, focusing on how they can provide clarity on what this negotiation period involves and what non commercially sensitive information can be shared with patient groups so they can update the patients they represent

Bridging the gap between the single technology assessment pathway and highly specialised technology pathway

- ⇒ NICE should ensure the upcoming review of the HST programme routing criteria includes consultation with the rare disease community on its appropriateness for the assessment of very rare diseases. It should also consider the introduction of guidelines for when the topic selection oversight panel should apply flexibility in the application of the criteria
- ⇒ NHS England should analyse the factors behind the under-utilisation of the Innovative Medicines Fund and consider whether reform to its criteria is needed to facilitate the approval of rare disease treatments that are candidates for managed access agreements. NHS England should also explore how it can be used to facilitate rapid access to new treatments post marketing authorisation, in line with NICE's work on rapid entry to managed access
- ⇒ Building on the commitment to deliver two innovative payment models for advanced therapy medicinal products made in the 2024 voluntary scheme for branded medicines pricing, access and growth, NHS England should consider whether additional commercial flexibilities should be introduced for rare disease medicines

Evaluating the impact of the severity modifier in enabling access to rare disease treatments

- ⇒ NICE's planned review of the impact of the severity modifier should include a specific analysis of the impact of the introduction of the modifier on the assessment of treatments for rare diseases, including data on how and where it has been utilised to date

Conclusion

The UK Rare Diseases Framework prioritises improving access to specialist care, treatments and drugs. Exciting progress has been made in some areas against this objective; scientific advancements now mean we have treatments available in rare conditions that previously had no treatment option. NICE and NHSE have enabled patient access to innovative new therapies from spinal muscular atrophy (SMA) to haemophilia B and inherited retinal disorders.

However, our engagement demonstrates the growing frustration from the rare disease community that NICE's current methods and processes are not suitable for the assessment of rare disease treatments and patients are missing out on access to potentially life changing therapies as a result. In particular, members expressed disappointment that the recent methods and processes review has not had a material impact on the likelihood of a rare disease treatment being recommended for use.

The SHCA welcomes the ongoing NICE review of the impact of its updated methods and processes, as well as the review of the HST criteria and severity modifier. Alongside this work, it is important that NICE studies the evidence base for genuine reform to the rare disease assessment pathway by commissioning primary research on the social value associated with treating rare diseases, utilising the NICE Listens programme. This will enable NICE to determine the public appetite for treating rare diseases differently to more common conditions and responding to the challenges set out in this report.

August 2024

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